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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Mercaptopurine (oral suspension) for the treatment of acute lymphoblastic leukaemia

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 30 April 2009, orphan designation (EU/3/09/628) was granted by the European Commission to Nova Laboratories Limited, United Kingdom, for mercaptopurine (oral suspension) for the treatment of acute lymphoblastic leukaemia.

What is acute lymphoblastic leukaemia?

Acute lymphoblastic leukaemia (ALL) is a cancer of the white blood cells called lymphocytes. In this disease, the lymphocytes multiply too quickly and live for too long, so there are too many of them circulating in the blood. These abnormal lymphocytes are not fully developed and do not work properly. Over a period of time, they replace the normal white cells and red blood cells and platelets in the bone marrow (the spongy tissue inside the large bones in the body).

ALL is the most common type of leukaemia in young children, but the disease also affects adults, especially those aged 65 years and older. Many people with ALL can be cured. However, despite the available treatments, ALL remains a serious and life-threatening disease in some patients.

What is the estimated number of patients affected by the condition?

At the time of designation, ALL affected approximately 1.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 61,000 people*, and is below the ceiling for orphan

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 504,800,000 (Eurostat 2009).

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designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

Treatment for ALL is complex and depends on a number of factors including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. At the time of designation, the main treatment of ALL was chemotherapy (medicines used to kill cancer cells) followed by or combined with radiotherapy (using radiations to kill cancer cells). Bone marrow transplantation was also used. This is a complex procedure where the bone marrow of the patient is destroyed and replaced with bone marrow from a matched donor.

The sponsor has provided sufficient information to show that mercaptopurine (oral suspension) might be of significant benefit for the patients because of the way in which the medicine is given. Mercaptopurine is already available in the European Union as tablets for the treatment of ALL. Mercaptopurine (oral suspension) is a liquid form of mercaptopurine, which could be given to children who have difficulty swallowing tablets. This may make it easier for children to take the medicine and allow the dose to be adjusted more precisely according to the patient's weight. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Mercaptopurine (oral suspension) is expected to work in the same way as mercaptopurine tablets. Mercaptopurine is an anticancer medicine that belongs to the group 'antimetabolites'. It works by interfering with the production of DNA within cells, preventing them from making more copies of their DNA. This means that cancer cells cannot divide and eventually die.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, a 'bioequivalence' study in adults had been planned, to find out whether the oral suspension of mercaptopurine was treated by the body in the same way as the tablets.

At the time of submission, mercaptopurine (oral suspension) was not authorised anywhere in the EU for ALL or designated as orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 March 2009 recommending the granting of this designation.

Update: mercaptopurine (oral suspension) (Xaluprine) has been authorised in the EU since 9 March 2012 for the treatment of acute lymphoblastic leukaemia (ALL) in adults, adolescents and children.

More information on Xaluprine can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Mercaptopurine (oral suspension)	Treatment of acute lymphoblastic leukaemia
Bulgarian	Меркаптопурин (перорална суспенсия)	Лечение на остра лимфобластна левкемия
Czech	Mercaptopurine (peronální suspense)	Léčba akutní lymfoblastické leukémie
Danish	Mercaptopurine (oral suspension)	Behandling af akut lymfoblastær leukæmi
Dutch	Mercaptopurine (suspensie voor oraal gebruik)	Behandeling van acute lymfoblastaire leukemie
Estonian	Merkaptopuriin (eroraalne suspensioon)	Ägeda lümfoblastilise leukeemia ravi
Finnish	Merkaptopuriini (oraalisuspensio)	Akuutin lymfoblastileukemian hoito
French	Mercaptopurine (suspension buvable)	Traitement de la leucémie lymphoblastique aiguë
German	Mercaptopurin (Suspension zum Einnehmen)	Behandlung der akuten lymphatischen Leukämie
Greek	Μερκαπτοπουρίνη (πόσιμο εναιώρημα)	Θεραπεία της οξείας λεμφοβλαστικής λευχαιμίας
Hungarian	Mercaptopurine (belsőleges szuszpenzió)	Akut lymphoblastos leukaemia kezelése
Italian	Mercaptopurina (sospensione orale)	Trattamento della leucemia linfoblastica acuta
Latvian	Merkaptopurīns (suspensija iekšķīgai lietošanai)	Akūtas limfoblastiskas leikozes ārstēšana
Lithuanian	Merkaptopurinas (geriamoji suspensija)	Ūmios limfoblastinės leukemijos gydymas
Maltese	Mercaptopurine (likwidu b'ħafna partiċelli żgħar tittieħed mill-ħalq)	Kura tal-lewkimja limfoblastika akuta
Polish	Mercaptopurine (zawiesina doustna)	Leczenie ostrej białaczki limfoblastycznej
Portuguese	Mercaptopurina (suspensão oral)	Tratamento da leucémia linfoblástica aguda
Romanian	Mercaptopurină (suspensie orală)	Tratamentul leucemiei limfoblastice acute
Slovak	Merkaptopurín (perorálna suspenzia)	Liečba akútnej lymfoblastickej leukémie
Slovenian	Merkaptopurine (peroralna suspensja)	Zdravljenje akutne limfoblastne levkemije
Spanish	Mercaptopurina (suspensión oral)	Tratamiento de la leucemia linfoblástica aguda
Swedish	Mercaptopurin (oral suspension)	Behandling av akut lymfatisk leukemi
Norwegian	Merkaptopurin (mikstur, suspensjon)	Behandling av akutt lymfoblastisk leukemi
Icelandic	Mercaptópúrín (mixture, dreifa)	Meðferð við bráðu eitilfrumuhvítblæði

[1] At the time of designation